Arrowhead Pharmaceuticals Initiates Expanded Access Program for Plozasiran and Announces Upcoming Presentation of Clinical Data

March 25, 2024

- Expanded Access Program initiated to make investigational plozasiran available to patients with familial chylomicronemia syndrome

- New SHASTA-2 Phase 2 clinical data in patients with severe hypertriglyceridemia to be presented at ACC.24

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 25, 2024-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it has initiated an Expanded Access Program (EAP) to make investigational plozasiran available outside of a clinical trial for patients with familial chylomicronemia syndrome (FCS) who meet certain program eligibility criteria. Arrowhead will also present new final Phase 2 clinical data from the double-blind portion of the SHASTA-2 study of plozasiran in a late-breaking oral presentation at the upcoming American College of Cardiology 73rd Annual Scientific Session & Expo (ACC.24), being held in Atlanta on April 6-8, 2024.

“Arrowhead is committed to supporting patients living with various lipid disorders and the physicians who treat them. To that end, we are pleased that Dr. Daniel Gaudet, Professor of Medicine at Université de Montréal and President and Scientific Director of ECOGENE-21, will present final data in a late-breaking oral presentation at ACC.24 from the SHASTA-2 study of plozasiran in patients with severe hypertriglyceridemia,” said Bruce Given, M.D., interim chief medical scientist at Arrowhead. “Our Phase 3 PALISADE study of plozasiran in patients with FCS is nearing completion and we are further supporting the patient community by initiating an expanded access program for patients and physicians interested in receiving treatment with plozasiran outside of a clinical trial. In addition, Arrowhead medical affairs representatives will be hosting a company booth at ACC.24 with resources for physicians to learn more about the mechanism of cardiometabolic diseases we are studying and our pipeline of RNAi therapeutics that leverage Arrowhead’s proprietary TRiM ™ platform.”

Arrowhead is committed to bringing new investigational medicines to patients with serious diseases as quickly and efficiently as possible. The company’s EAP is a potential pathway for patients with serious or immediately life-threatening diseases or conditions to gain access to an investigational medical product for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

The plozasiran EAP is for individuals living with FCS. As with any investigational medicine that has not been approved by regulatory authorities, investigational plozasiran may or may not be effective in treating your diagnosis or condition, and there may be risks associated with its use. If you are a patient or caregiver wishing to know more about this plozasiran EAP for FCS, please discuss this EAP and all treatment options with your treating physician. If you are a treating physician and are seeking information about the plozasiran EAP or would like to request access for a patient, please contact EAP@arrowheadpharma.com.

Details about the ACC.24 presentation are listed below.

American College of Cardiology 73rd Annual Scientific Session & Expo – April 6-8, 2024

Title: **Plozasiran (ARO-APOC3), An Investigational RNAi Therapeutic, Demonstrates Profound and Durable Reductions in APOC-3 and Triglycerides (TG) in Patients With Severe Hypertriglyceridemia (SHTG), SHASTA-2 Final Results**
Date/Time: April 7, 2024, 9:00 a.m. EDT
Presenter: Daniel Gaudet MD, PhD
Session: Late-Breaking Clinical Trials II

Presentation materials may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website after the oral presentation concludes.

About Plozasiran

Plozasiran, previously called ARO-APOC3, is a first-in-class investigational RNA interference (RNAi) therapeutic designed to reduce production of Apolipoprotein C-III (APOC3) which is a component of triglyceride rich lipoproteins (TRLs) and a key regulator of triglyceride metabolism. APOC3 increases triglyceride levels in the blood by inhibiting breakdown of TRLs by lipoprotein lipase and uptake of TRL remnants by hepatic receptors in the liver. The goal of treatment with plozasiran is to reduce the level of APOC3, thereby reducing triglycerides and restoring lipids to more normal levels.

In multiple clinical studies, investigational plozasiran demonstrated reductions in triglycerides and multiple atherogenic lipoproteins in patients with familial chylomicronemia syndrome (FCS), severe hypertriglyceridemia (SHTG), and mixed dyslipidemia (MD). Plozasiran has demonstrated a favorable safety profile to date with treatment emergent adverse events reported that reflect the comorbidities and underlying conditions of the study populations. Plozasiran is currently being investigated in the Phase 3 PALISADE clinical study in patients with FCS, which is on schedule to be completed in the middle of 2024. Phase 2 studies in patients with SHTG and MD, SHASTA-2 and MUIR respectively, are complete and additional Phase 3 studies are planned to begin shortly.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, Arrowhead therapies trigger the RNA interference mechanism to induce rapid, deep, and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead’s RNAi-based therapeutics leverage this natural pathway of gene silencing.

For more information, please visit www.arrowheadpharma.com, or follow us on X (formerly Twitter) at @ArrowheadPharma or on LinkedIn. To be added to the Company's email list and receive news directly, please visit http://ir.arrowheadpharma.com/email-alerts.

Safe Harbor Statement under the Private Securities Litigation Reform Act:

This news release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform

Act of 1995. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "hope," "intend," "plan," "project," "could," "estimate," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects or benefits of our collaborations with other companies, or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. These statements are based upon our current expectations and speak only as of the date hereof. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of numerous factors and uncertainties, including the impact of the ongoing COVID-19 pandemic on our business, the safety and efficacy of our product candidates, decisions of regulatory authorities and the timing thereof, the duration and impact of regulatory delays in our clinical programs, our ability to finance our operations, the likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize drug candidates, the timing for starting and completing clinical trials, rapid technological change in our markets, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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